In contrast, there are strong differences in the reimbursement of orphan drugs in Nordic and devolved markets. Relevant stakeholders in reimbursement assessment are national HTA (health technology assessment) agencies or regional authorities. In all archetypes, except health insurance markets, regional authorities are the relevant funding stakeholders. **CONCLUSIONS:** There are several similarities and differences in orphan drug reimbursement across European countries. While some countries do not differentiate between orphan and non-orphan drugs in decision-making or funding, others have implemented specific policies.

PSY109

REIMBURSED PRICE OF ORPHAN DRUGS: A VALUE BASED FRAMEWORK

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OBJECTIVES: The present work reviews current practices for the definition of the reimbursed price of orphan drugs, and proposes a conceptual framework for their value-based pricing along with a roadmap for its possible implementation. **METHODS:** Based on a literature analysis, we systematize the current discussions on the topic within a conceptual framework intended to support evaluations and decision-making for determining a value-based reimbursed price for orphan drugs. **RESULTS:** Our analysis points out the "black box" pricing mechanism of orphan drugs and the limited consistency of some of the constituent domains of Health Technology Assessment when applied to orphan medical products (safety, clinical effectiveness, costs and economic evaluation, ethical analysis, and organizational and social aspects). The proposed framework comprises: a) elements of societal value and methods for its assessment, b) exchanges of valuable and trustworthy information between relevant stakeholders from an early stage, c) innovative reimbursement approaches to balance the need for evidence-based decisions with timely access to innovative drugs for patients with rare diseases, d) societal participation in the risky entrepreneurship of producing orphan drugs. Considering the areas reported in the proposed framework, we identified a possible roadmap for its implementation via three critical phases: i) sharing available experiences, ii) integrating and systematizing methods for appropriate use, iii) identifying delegate agencies. **CONCLUSIONS:** Additional piloting of emerging experiences and sharing of implementations developed worldwide are needed, along with the identification of an internationally agreed-upon taxonomy and pan-national recognised subjects to be delegated part of the HTA activities currently spread over a multiplicity of subjects.

PSY110

HORIZONTAL VS. VERTICAL EQUITY: MARKET ACCESS OF ORPHAN DRUGS IN FRANCE AND UK

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OBJECTIVES: In 2000 EU introduced an orphan legislation in order to stimulate the development of drugs for rare diseases. Nevertheless, the actual availability of orphan drugs (ODs) in the EU depends on national authorities. The study aims to compare OD availability and market access in France and the UK. **METHODS:** The details on OD prices and reimbursement were extracted from Ameli database in France and British National Formulary in the UK. The molecules were considered available if the price were published. NICE recommendations were extracted from the official website. **RESULTS:** 55 and 56 ODs were available in the UK and France, respectively, among 82 ODs authorized in the EU. 50 ODs were available in the two countries. Most of ODs sold in pharmacy were 100% or 65% reimbursed in France. All drugs are 100% reimbursed in the UK and when used in hospital in France. Only 20 ODs were assessed by the NICE in 24 indications. In 10 cases NICE did not recommend the drug, in five cases the indication was restricted. For more than 60% of molecules prices were higher in the UK. The median difference was about 10%. **CONCLUSIONS:** The number of commercialized ODs is similar in the two countries. However, given the fact that most of ODs has not been recommended by the NICE, it is unlikely that they will be prescribed. Prices are free of control in the UK leading to higher prices compared with France. Although prices are published in the both countries, the real prices are impacted by implementing patient access schemes in the UK and hidden volume agreements in France. Interestingly, while cost-effectiveness assessment is becoming mandatory in France, NICE introduces a new methodology for OD moving away from economic evaluation.

PSY111

ORPHAN DRUG REGULATION IN THE USA, EUROPEAN UNION, JAPAN AND SOUTH KOREA: A COMPARATIVE ANALYSIS

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OBJECTIVES: The study aims to review and compare orphan drug (OD) policies in the USA, EU, Japan and South Korea. **METHODS:** The OD policies (regulation and list of incentives) were extracted from the official websites of the regulatory authorities. **RESULTS:** USA was the first to implemented OD legislation in 1983, followed by Japan (1993), South Korea (1997), and the EU (2000). The prevalence threshold is the highest in the USA and the lowest in Japan. The rarity of the disease or the lack of profitability is the only criteria for granting orphan status in the USA. In the EU, Japan and South Korea high unmet needs should be demonstrated. Some non-rare conditions are eligible for orphan designation in Japan and South Korea. Development feasibility needs to be proven in Japan. Specific scientific assistance is available in all regions except South Korea. Market exclusivity is granted for 10 years in the EU and 7 years in the USA. Financial assistance through fees reduction is available in all geographies. All regions but the EU also provide fast track marketing authorization procedure. Tax credits and/or tax reduction for clinical development and special grant programs are available in the USA and Japan. Only in South Korea, products that have already obtained orphan designation in elsewhere can benefit from a partial exemption from dossier submission for orphan designation and market authorization. **CONCLUSIONS:** OD regulation and incentives are similar by nature but differ in magnitude. It is unclear how the profitability criteria are applied in US as multiple orphan products are blockbuster. USA applies the mildest criteria for granting orphan status and provides the greatest financial support. The financial assistance is the most modest in the EU while exclusivity is the longest. EU adds an additional layer at member state level.

PSY112

ECONOMIC IMPACT OF THE END OF THE MARKET EXCLUSIVITY FOR ORPHAN DRUGS

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OBJECTIVES: In order to stimulate the research and development of orphan drugs, in 2000 the EU introduced new legislation with the aim of providing incentives for the development of drugs for rare disorders. One of the strongest incentives, regarding experience in the United States of America and Japan, is a market exclusivity period post authorization for designated products. The objective of this study is to assess if the 10 year market exclusivity has created a market failure by assessing the prices and the competition environment of drugs for which this period has come to an end. **METHODS:** From the 2014 Orphanet's list, we retrieved the 20 drugs that have lost their market exclusivity for at least one indication (26 indications). It is worth to note that some of them still have market exclusivity for another indication, which means they may still be on the orphan drug register. We focused on the French market and used French administrated prices. **RESULTS:** 63% (12 drugs) of the sample have not suffered a drop in prices after the market exclusivity loss. Among all the studied variables, the most important event to trigger a price drop seems to be an extension of indication. 40% (10 drugs) of the sample were able to see the arrival of competitors during their period of market exclusivity even if they target a small population. **CONCLUSIONS:** These results show that the market exclusivity does not necessarily create an inflationary effect and a monopoly for orphan drugs. Market exclusivity would act as a protectionist measure. Its major interest is in the beginning of the product's life and for old drugs for which the molecule is not under the patent at the time of the approval.

PSY113

VALUE ASSESSMENT AND PRICING FRAMEWORKS FOR RARE DISEASE TREATMENTS: NEW APPROACHES FROM THE LITERATURE

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OBJECTIVES: The use of cost effectiveness analysis for treatments for rare diseases has proved difficult and alternative frameworks for assessing value and determining pricing and reimbursement have been sought. Here we summarise rare disease specific methodologies proposed in the literature. **METHODS:** A systematic literature review of Medline and EMBASE databases was conducted for the period 2000 – 2014 without geographic restriction. The search sought to identify papers that proposed specific frameworks for a) assessing the value of rare disease treatments or b) determining the price or reimbursement status of such drugs. Policy papers, commentaries, and review articles were included. Clinical or economic studies of specific rare diseases and their treatments were excluded. **RESULTS:** The literature review identified 1,034 papers. Eleven studies proposed specific methods for assessing rare disease treatments. The most commonly proposed approach (7 of 11 papers) involved multi-criteria decision analysis. Of these studies, 5 proposed MCDA frameworks; one study applied and validated MCDA frameworks with rare disease stakeholders and another study investigated the relationship between MCDA domain attributes and the cost of rare disease drugs. Of non-MCDA methods, one study described a novel decision making framework that balances payer value factors with opportunity cost, and another discussed a policy framework for funding rare disease treatments in Ontario. Two studies specified non-value based pricing frameworks: one cost-based pricing and another 'grant and access' pricing. Limitations included challenges identifying representative societal preferences, determining the perspective from which value should be assessed, and resistance to transparency from decision makers. **CONCLUSIONS:** The need for a new framework for the evaluation of rare disease treatments has been commonly recognised in the published literature. A number of authors have proposed alternative approaches to cost effectiveness, but practical challenges are limiting their application currently.

PSY114

RECOMMENDATIONS AND REIMBURSEMENT STATUS OF ORPHAN DRUGS IN UE COUNTRIES

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OBJECTIVES: The aim of this study was to review and compare decisions of particular European HTA Agencies as well as orphan drugs' reimbursement status in corresponding countries. **METHODS:** 93 orphan drugs, approved in Europe, were identified. We considered the following European HTA Agencies: AOTM (Poland), NICE (England), AWMSG (Wales), HAS (France), SMC (Scotland), IQWIG (Germany). Data on the Agencies' recommendations (positive, negative, conditional) and reimbursement status were collected for each drug. **RESULTS:** Among all identified orphan drugs 18%-60% were assessed by the HTA Agencies in particular countries. AWMSG assessed 56 orphans, and IQWIG only 17 drugs. Among 93 orphan drugs 23 (25%) have never been assessed by any of analyzed European HTA Agencies. The average rates of positive, negative and conditional recommendations among included Agencies were 51.6%, 39.4% and 9.0%, respectively. The highest rate of positive recommendations was obtained by HAS Agency (France) which was 89.4%, and the lowest by AOTM (Poland) - 17.9%, but AOTM has issued also 42.9% conditional recommendations. On average, 13% of approved orphan drugs are reimbursed